

### Enhancing Facilities for Genetic Manipulation and Engineering of Human Embryonic Stem Cells at UCSD

## **Grant Award Details**

Enhancing Facilities for Genetic Manipulation and Engineering of Human Embryonic Stem Cells at UCSD

Grant Type: Shared Labs

Grant Number: CL1-00522-1.1

Investigator:

Name: Karl Willert

**Institution**: University of California, San Diego

Type: PI

**Award Value:** \$1,728,080

Status: Closed

#### **Grant Application Details**

Application Title: Enhancing Facilities for Genetic Manipulation and Engineering of Human Embryonic Stem Cells

#### **Public Abstract:**

Human embryonic stem cell (hESC) research promises to be of fundamental importance in the study and treatment of various human diseases, including cancer, neurodegenerative disorders and organ failure. In recent years we have made great strides in advancing hESC research as documented by the large number of successful, high-impact laboratories and breadth of research projects here. In addition, we are situated among several other first-rate institutions, all of which have joined in an unparalleled research environment for hESC research.

Since the creation of the California Institute for Regenerative Medicine, we have devoted both space and financial resources to promote hESC research. Our institutional commitment has as a cornerstone the creation of a core facility for hESC research to foster and promote hESC research at this and surrounding institutions. To date the facility has served to (1) train scientists in the basic methodologies to conduct hESC research (2) facilitate hESC research for many investigators, both established and beginning scientists, and (3) provide a "safe haven" that is sheltered from any federal funding sources thus allowing unimpeded hESC research. However, due to the high demand on space, equipment and technologies, the present facilities are insufficient to sustain the ongoing and proposed research projects.

We therefore request funding from CIRM to expand this facility and enhance its scientific output and creativity. In addition to providing expanded adequate facilities for our many scientists and clinicians embarking on hESC research, our major scientific goals for the shared research laboratory are (1) the development of protocols for the generation of genetically marked HESC lines, (2) the improvement of protocols for derivation of mature cell types, with an emphasis on neural differentiation, and (3) the development of novel surfaces and materials for the large scale growth and production of hESCs. These goals synergize the expertise of several departments, including the departments of Bioengineering, Materials Science, Biological Sciences, Pharmaceutical Sciences and the School of Medicine.

The support provided by this shared research grant will allow our institution to enhance our interdisciplinary stem cell program so that we may accelerate our goals of improving health and conquering diseases through regenerative medicine.

# Statement of Benefit to California:

Human embryonic stem cells (hESC) provide the "raw material" that can potentially provide mature cell types for developing new disease therapies. Understanding how to control the growth and differentiation of hESCs, however, requires extensive research. Unfortunately, federal restrictions limit progress.

In 2005, our institution established a shared research laboratory which has provided essential training and has made space and technologies available to conduct hESC research without federal restrictions. However, the needs of researchers are beginning to exceed our limited resources. CIRM funding will allow expansion of the existing facility and the development of key technologies essential to ongoing and proposed projects at this and other institutions throughout California.

The proposed expansion includes creation of a satellite with emphasis on bioengineering technologies needed to develop therapeutic delivery vehicles and grow tissue engineered implants from hESC-derived cells. This satellite will leverage our international leadership in cell and tissue engineering and significant experience in translational research and technology transfer. The unique strength of interdisciplinary partnerships will accelerate translation of new scientific discoveries to clinical practice and new therapeutic agents that will benefit California's healthcare system and global competitiveness.

CIRM funding will be enhanced by the investment already made by our institution in hESC research. The proposal integrates scientific need with the proposed expansion in cell biology and engineering abilities. Our request comes in direct response to needs of productive, experienced researchers in a context where professors, doctors, ethicists, social and political scientists can contribute to advancements in service of human health. Our context provides the teaching environment that will help engage California's best young minds, and impart the latest discoveries to our students.

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